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## Combination therapy to Enhance Antisense Mediated Exon Skipping for Duchenne Muscular Dystrophy

### Grant Award Details

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Combination therapy to Enhance Antisense Mediated Exon Skipping for Duchenne Muscular Dystrophy

**Grant Type:** Disease Team Therapy Planning I

**Grant Number:** DR2-05426

**Investigator:**

<b>Name:</b>	Stanley Nelson
<b>Institution:</b>	University of California, Los Angeles
<b>Type:</b>	PI

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**Disease Focus:** Muscular Dystrophy, Skeletal/Smooth Muscle disorders

**Award Value:** \$68,947

**Status:** Closed

### Progress Reports

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**Reporting Period:** Year 1

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### Grant Application Details

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**Application Title:** Combination therapy to Enhance Antisense Mediated Exon Skipping for Duchenne Muscular Dystrophy

**Public Abstract:**

A drug was identified through the use of muscle stem cells that can enhance the effectiveness of exon skipping by antisense oligonucleotides to the DMD gene to restore dystrophin expression and at least partially correct the defect responsible for loss of muscle function in Duchenne. We propose to test the effectiveness of this drug in combination with antisense oligonucleotides as a novel therapeutic strategy for Duchenne muscular dystrophy (DMD). DMD is the most common muscular dystrophy and leads to progressive muscle loss in boys resulting in severe weakness, and is caused by mutations in the DMD gene. DMD generally leads to death in the teens or early 20's, making Duchenne one of the most severe disorders in humans. Further, Duchenne occurs in 1/3500 boys, making it one of the most common genetic disorders. There are no highly effective therapies. Thus, there is an urgent need to develop new and highly effective therapies. We propose to perform the necessary studies using DMD patient-derived iPS and animal models to perform safety studies that will permit regulatory approval to test the safety and efficacy of the combination therapy in Duchenne muscular dystrophy. The goal of the treatment is to make a functional dystrophin protein the patient's body by altering the RNA in each muscle cell. Preliminary results indicate that the process is relevant to about 70% of those afflicted by Duchenne.

**Statement of Benefit to California:**

Since Duchenne muscular dystrophy is the most common lethal genetic disorder, there are over 1,000 patients affected in the state of California alone, 15,000 nationwide, and 300,000 worldwide. Duchenne muscular dystrophy has a large direct economic impact with intensive medical care with substantial disability. There is an obvious huge impact on the family as well. More effective therapies will directly benefit these families, lead to increased productivity. Further, a California based company will have developed a key therapy for an otherwise lethal genetic disorder further demonstrating California's leadership in medical science, and generating novel business opportunities within the Biotechnology industry in California.

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